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## Assessment of Safety and Functional Efficacy of Stem Cell-Based Therapeutic Approaches Using Retinal Degenerative Animal Models.

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**Funding Grants:** Restoring vision by sheet transplants of retinal progenitors and retinal pigment epithelium (RPE) derived from human embryonic stem cells (hESCs)

### Public Summary:

This paper is a review of stem cell therapies in different animal models. Dysfunction and death of retinal pigment epithelium (RPE) and or photoreceptors can lead to irreversible vision loss. The eye represents an ideal microenvironment for stem cell-based therapy because it is considered an "immune privileged" site, and the number of cells needed for therapy is relatively low for the area of focused vision (macula). Further, surgical placement of stem cell-derived grafts (RPE, retinal progenitors, and photoreceptor precursors) into the vitreous cavity or subretinal space has been well established. For preclinical tests, assessments of stem cell-derived graft survival and functionality are conducted in animal models by various noninvasive approaches and imaging modalities. In vivo experiments conducted in animal models based on replacing photoreceptors and/or RPE cells have shown survival and functionality of the transplanted cells, rescue of the host retina, and improvement of visual function. Based on the positive results obtained from these animal experiments, human clinical trials are being initiated. Despite such progress in stem cell research, ethical, regulatory, safety, and technical difficulties still remain a challenge for the transformation of this technique into a standard clinical approach. In this review, the current status of preclinical safety and efficacy studies for retinal cell replacement therapies conducted in animal models will be discussed.

### Scientific Abstract:

Dysfunction and death of retinal pigment epithelium (RPE) and or photoreceptors can lead to irreversible vision loss. The eye represents an ideal microenvironment for stem cell-based therapy. It is considered an "immune privileged" site, and the number of cells needed for therapy is relatively low for the area of focused vision (macula). Further, surgical placement of stem cell-derived grafts (RPE, retinal progenitors, and photoreceptor precursors) into the vitreous cavity or subretinal space has been well established. For preclinical tests, assessments of stem cell-derived graft survival and functionality are conducted in animal models by various noninvasive approaches and imaging modalities. In vivo experiments conducted in animal models based on replacing photoreceptors and/or RPE cells have shown survival and functionality of the transplanted cells, rescue of the host retina, and improvement of visual function. Based on the positive results obtained from these animal experiments, human clinical trials are being initiated. Despite such progress in stem cell research, ethical, regulatory, safety, and technical difficulties still remain a challenge for the transformation of this technique into a standard clinical approach. In this review, the current status of preclinical safety and efficacy studies for retinal cell replacement therapies conducted in animal models will be discussed.

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